

Clinical Trials

The STEER OAV101B12301 Study

[Study passport](#)

 NOVARTIS | Reimagining Medicine

Welcome to the STEER Study!

This passport will help you keep track of your study tests and visits.

You will receive a stamp for each visit you complete.

Study team contacts

Name: _____

Phone: _____

Email: _____

Name: _____

Phone: _____

Email: _____

Being in the study is your choice and you can leave at any time.

This is me! _____

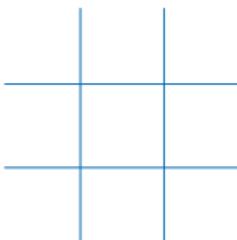
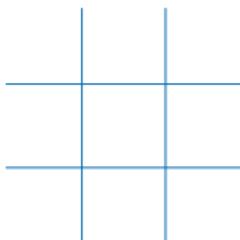
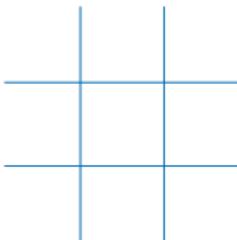
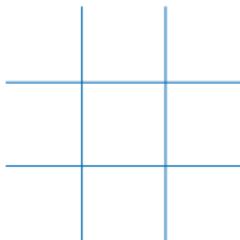
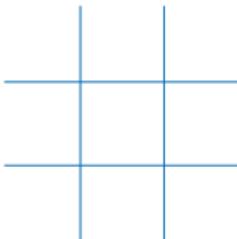
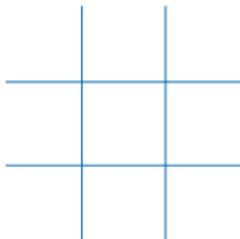
Draw a picture of yourself

My flag _____

Draw your country's flag

Date I started the study: _____

Tic-tac-toe:



The STEER Study

You will be in the STEER Study for up to 17 months and have about 29 doctor visits for health checks and tests. You will also stay overnight in the hospital two times. Your parent or guardian can stay with you.

Screening

Up to 60 days | At least 2 visits

Baseline (study start)

1 day before treatment | 1 visit

Treatment Period 1

Hospital stay

Follow-up Period 1

52 weeks (1 year) | 18 visits

Treatment Period 2

Hospital stay

Follow-up Period 2

12 weeks (3 months) | 8 visits

Screening At least 2 visits

Before you can start the study, your parents will sign an **Informed Consent Form** to give their permission for you to join. You may also sign an **Informed Assent Form** if you agree.

The doctor will then ask questions about your health and run tests to make sure the study is right for you.



Blood test



Body mass



Breathing test



Checkup



Head measurement



Health history



Heart activity



Heart imaging



Height



Medicine check



Mood check



Movement ability



Neurological examination



Swallowing test



Urine test



Vital signs



Weight



X-ray

You will not have all tests at both visits.

STAMP

STAMP

Baseline visit **Study start**



Blood test



Body mass



Breathing test



Checkup



Head measurement



Heart activity



Height



Medicine check



Movement ability



Movement monitoring*



Nasal swab



Neurological
examination



Saliva test



Stool test



Swallowing
test



Symptom
score



Urine test



Vital signs



Weight

*Only for some participants. This can be done at home.

STAMP

Treatment

This study has 2 treatment periods. For each, you will stay in the hospital for a few days.

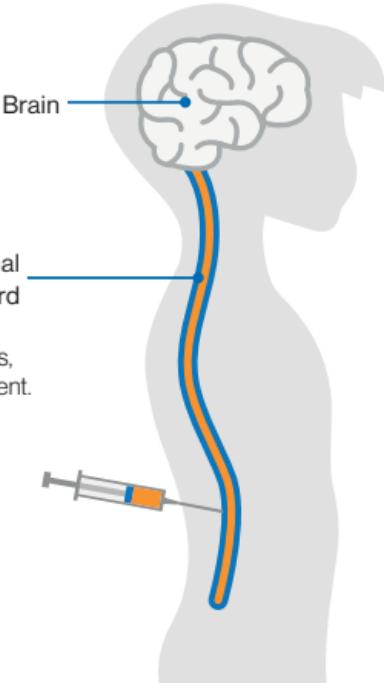
Part 1

During Part 1 of treatment, you will be placed in a group by chance to get either:

One-time injection of the study treatment (OAV101) into your lower back

or

A needlestick to the skin of your lower back (no medicine).



Part 2

After about 1 year of study visits, you may enter Part 2 of treatment.

If you had the study treatment injection during Part 1, you will now get a needlestick.

or

If you had a needlestick during Part 1, you will now get the study treatment injection.

Treatment 1 Hospital stay



Blood test



Checkup



Medicine check



Nasal swab



Neurological examination



Saliva test



Spinal tap



Stool test



Urine test



Vital signs



Weight

You will also be checked to see if the treatment is making you feel sick.

STAMP

STAMP

STAMP

Follow-up 1 18 visits



Blood test



Body mass



Breathing test



Checkup



Heart activity



Heart imaging



Height



Medicine check



Mood check



Movement ability



Movement monitoring*



Nasal swab



Neurological examination



Saliva test



Spinal muscular atrophy (SMA) score



Stool test



Swallowing test



Symptom score



Urine test



Vital signs



Weight



X-ray**

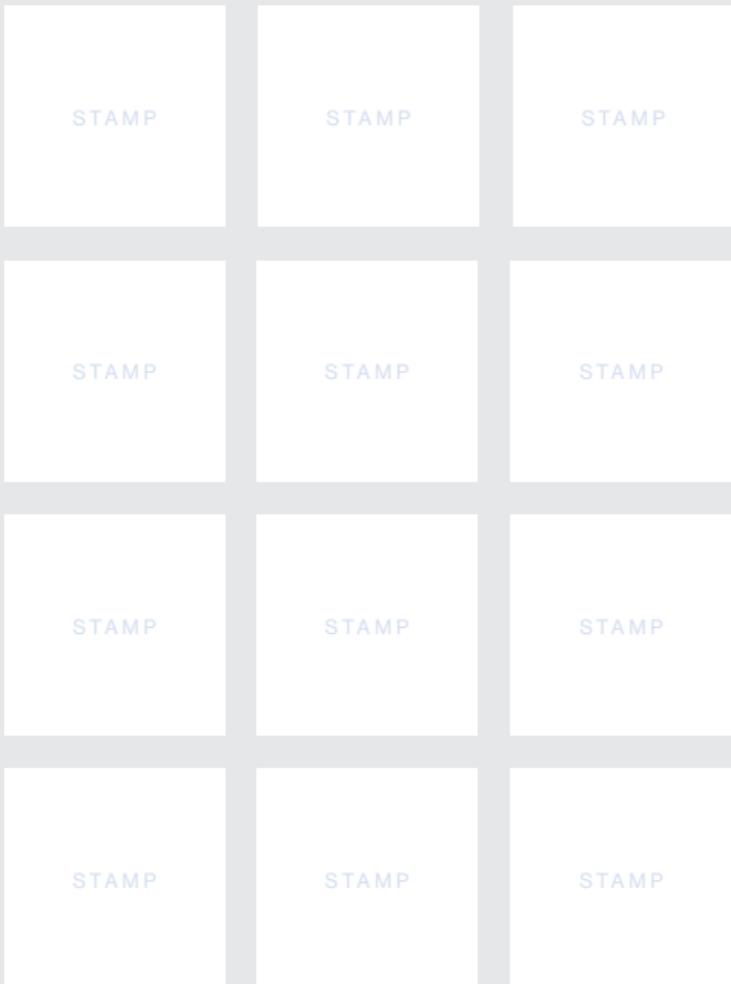
*Only for some participants. This can be done at home.

**Final visit only.

You will not have all tests at both visits.

You will also be checked to see if the treatment is making you feel sick.

Follow-up 1 18 visits





Treatment 2 Hospital stay



Body mass



Checkup



Medicine check



Nasal swab



Neurological
examination



Saliva test



Spinal tap



Stool test



Urine test



Vital signs



Weight

You will also be checked to see if the treatment is making you feel sick.

STAMP

STAMP

STAMP

Follow-up 2 8 visits



Blood test



Body mass



Breathing test*



Checkup



Heart activity



Heart imaging



Height



Medicine check



Mood check



Movement ability



Movement monitoring**



Nasal swab



Neurological examination



Saliva test



Spinal muscular atrophy (SMA) score

*Final visit only.

**Only for some participants. This can be done at home.

Follow-up 2 8 visits

(continued)



Stool test



Swallowing test



Symptom score



Urine test



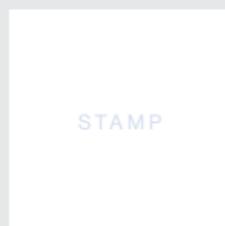
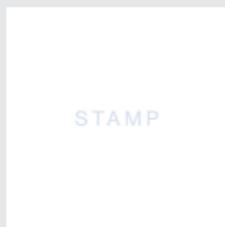
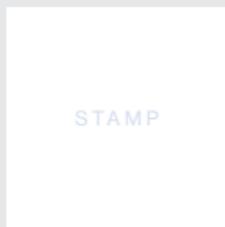
Vital signs



Weight

You will not have all tests at both visits.

You will also be checked to see if the treatment is making you feel sick.



Glossary:



Blood test: Small samples of blood will be taken to look at how much study medicine is in your body and what it may be doing inside your body. Girls who have started their periods will also have their blood tested for pregnancy.



Body mass: The doctor will calculate how much body fat you have based on your height and weight.



Breathing test: You will breathe into a tube attached to a computer to measure how much air you can breathe in and out of your lungs and how fast you can blow air out of your lungs.



Checkup: The doctor will check different parts of your body, such as your heart, lungs, muscles, and tummy.



Head measurement: The study team will wrap a flexible measuring tape around your head to see how big it is.



Health history: The doctor will ask questions about your health, your spinal muscular atrophy (SMA) and any surgeries you may have had.



Heart activity: A simple test called an electrocardiogram (ECG) will be used to check the electrical activity (rhythm) of your heart. For this test, you will lie down and small, sticky pads will be attached to your skin. The pads are connected with wires to a computer.



Heart imaging: A painless test called an echocardiogram uses sound waves to see your heart pumping and beating inside your body.



Height: The study doctor will use a measuring tape to measure the length of your lower leg (from your knee to your ankle) to calculate how tall you are.



Medicine check: The study doctor will ask questions about medicines you are taking now or have taken in the past.



Mood check: You will answer some questions about your mood and if you are feeling sad.



Movement ability: The study team will look at how well you can perform certain movements. They will give you scores based on your physical abilities.



Movement monitoring: Some participants will be selected to wear a movement monitor around their wrist while at home to keep track of their movement.



Nasal swab: The study doctor will collect a sample by gently rubbing the inside of your nose with a cotton swab.



Neurological examination: The study doctor will check to see how well different parts of your body are able to feel.



Saliva test: You will spit into a cup and the sample will be tested to see what the treatment may be doing inside your body.



Spinal muscular atrophy (SMA) score: The study team will use a scoring system to rate if and how your spinal muscular atrophy (SMA) is improving.



Spinal tap: A small amount of the fluid around your spine will be removed using a thin needle. The spinal fluid will be tested to see how your body may be responding to treatment.



Stool test: A small sample of your poo will be collected and tested to see what the treatment may be doing inside your body.



Swallowing test: A specialist will look to see how well you are able to eat and swallow.



Symptom score: The study team will use a scoring system to rate your symptoms of spinal muscular atrophy (SMA) and how they affect your ability to do everyday tasks.



Urine test: You will pee into a cup and your urine (pee) will be tested for certain things, such as sugar, protein and blood. Girls who have started their periods will also have their urine tested for pregnancy.



Vital signs: The study doctor will check your temperature, count how many breaths you take per minute, count how many times your heart beats per minute, and take your blood pressure to see how your heart is pumping.



Weight: The study doctor will weigh you on a scale without your shoes while you are wearing light clothing. Someone will help you to stand on the scale and hold you while you are weighed.



X-ray: An x-ray is used to take black-and-white pictures of the inside of your body. You will have an x-ray of your chest and back.

Notes:

Novartis - The STEER Study - Passport - 31-March-2022 -
English (Malaysia) - V1.0_Translated 3 May 2022
STEER-PP-En